Figure 1A

pBHG11Δpol

dl7001

Cotransfection into B-6 cells (E1+,pol+)
yielded:

AdΔpol/pBHG11

AdΔpol/dl7001
"AdΔpol"

= Δpol deletion
= dl7001-E3 deletions
= pBHG11-E3 deletions
= Packaging signal
S = ScaI sites
✗ = recombination site

Figure 1B

43 mu

del E3

pA-Lac Z-CMV 9 mu

Δpol

S S S XbaI S

BspHI

0-1 mu   pA-Lac Z-CMV

ClaI XbaI

pAdCMV/
LacZ/Δpol

dl7001-TP

del E3

Δpol

pA-Lac Z-CMV

AdLacZΔpol

X = recombination site   S= ScaI site

= TP

Figure 1C

Figure 2

~25 kb

AdLacZΔpol

MOI~50   MOI~500

48 24 10 0   48 24 10 0

Adsub360LacZ

MOI~5   MOI~50

48 24 10 0   48 24 10 0

hpi: →

Figure 3

Figure 4

Figure 5

B-6 cell infected cell lysates

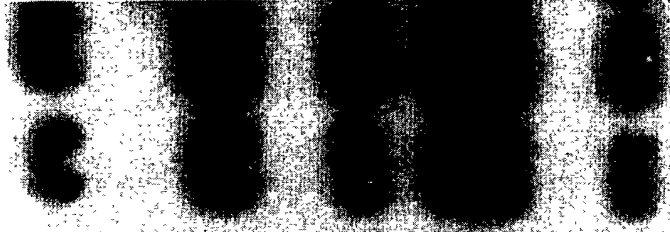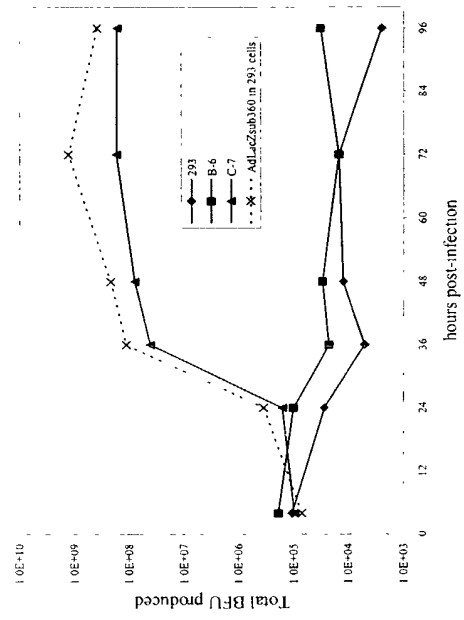1/100,000   1/10,000   1/1,000   1/10   1.0

Fiber

Figure 6

Figure 7

Fiber→

MOI=500

Figure 8

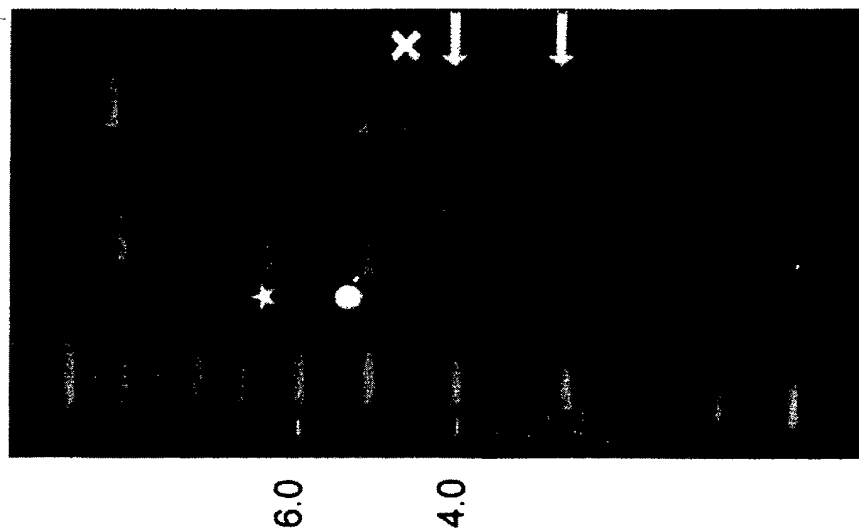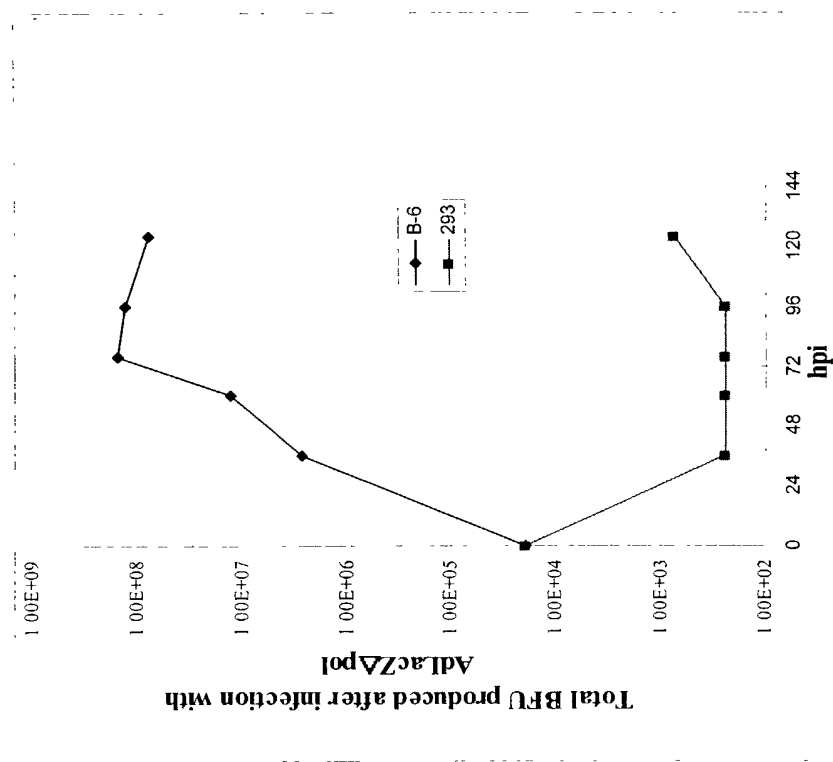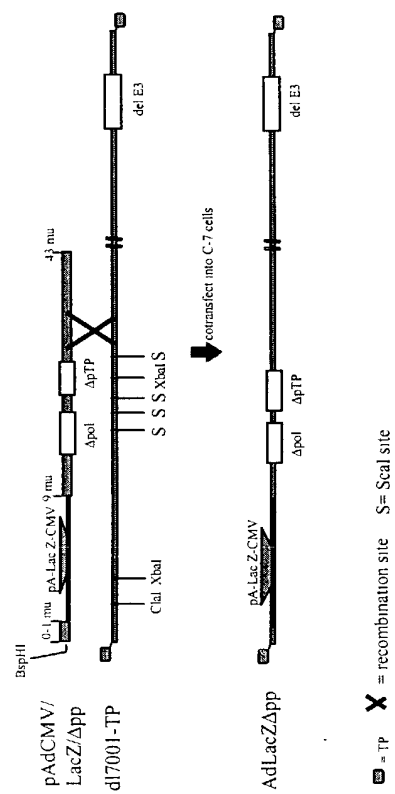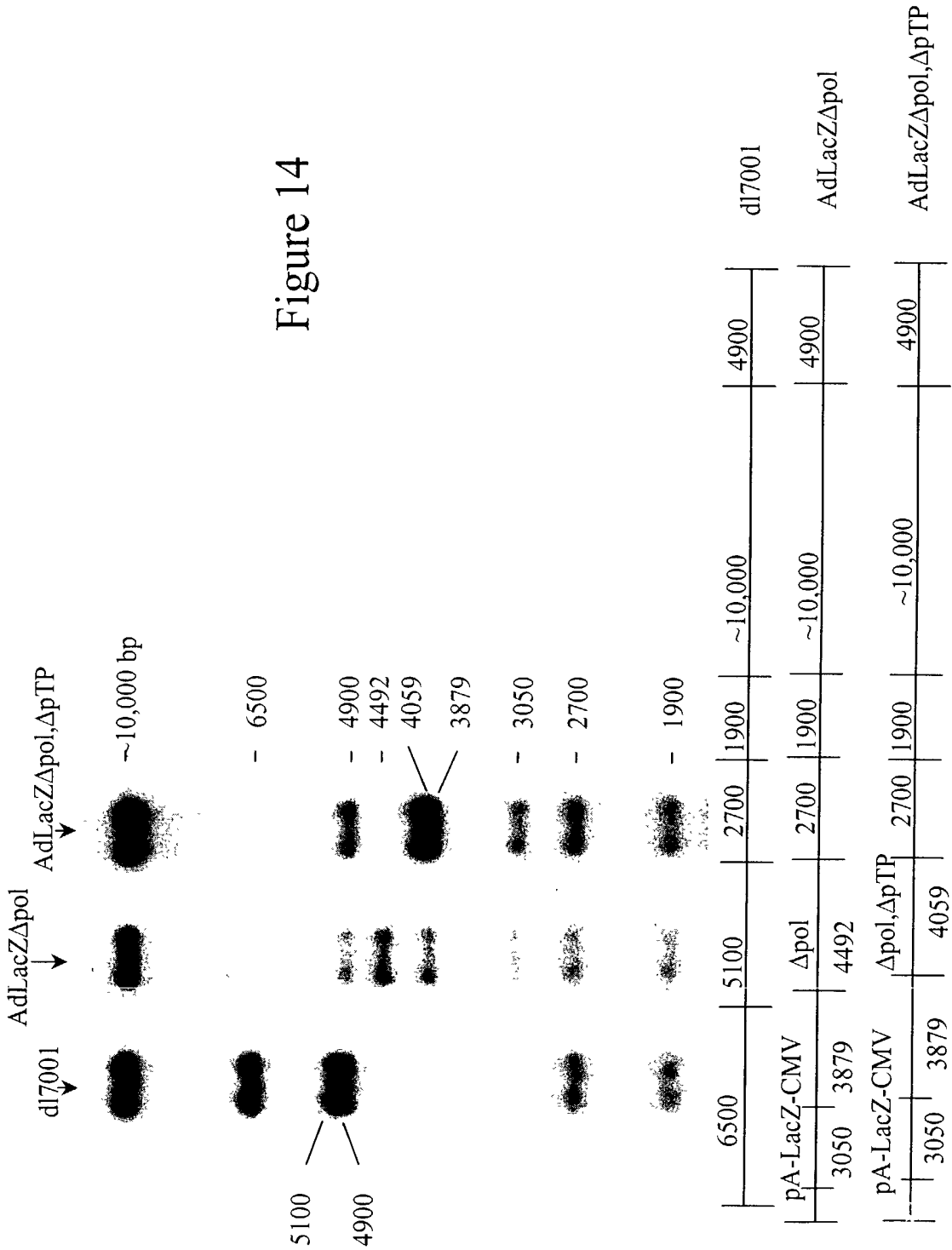Figure 9

Figure 10

Figure 11

Figure 12

pAdCMV/
LacZ/Δpp

BspH I

0-1 mu   pA-Lac Z-CMV 9 mu   4) mu

dl7001-TP

ΔpoI   ΔpTP

ClaI XbaI   S S S XbaI S   del E3

cotransfect into C-7 cells

AdLacZΔpp

pA-Lac Z-CMV   ΔpoI   ΔpTP   del E3

▢ = TP   ✗ = recombination site   S= ScaI site

Figure 13

Figure 14

Figure 15

- = Tripartite leaders
- = MLP-intron 1
- = transgene insertion/ E1 deletion site
- = VA RNA genes
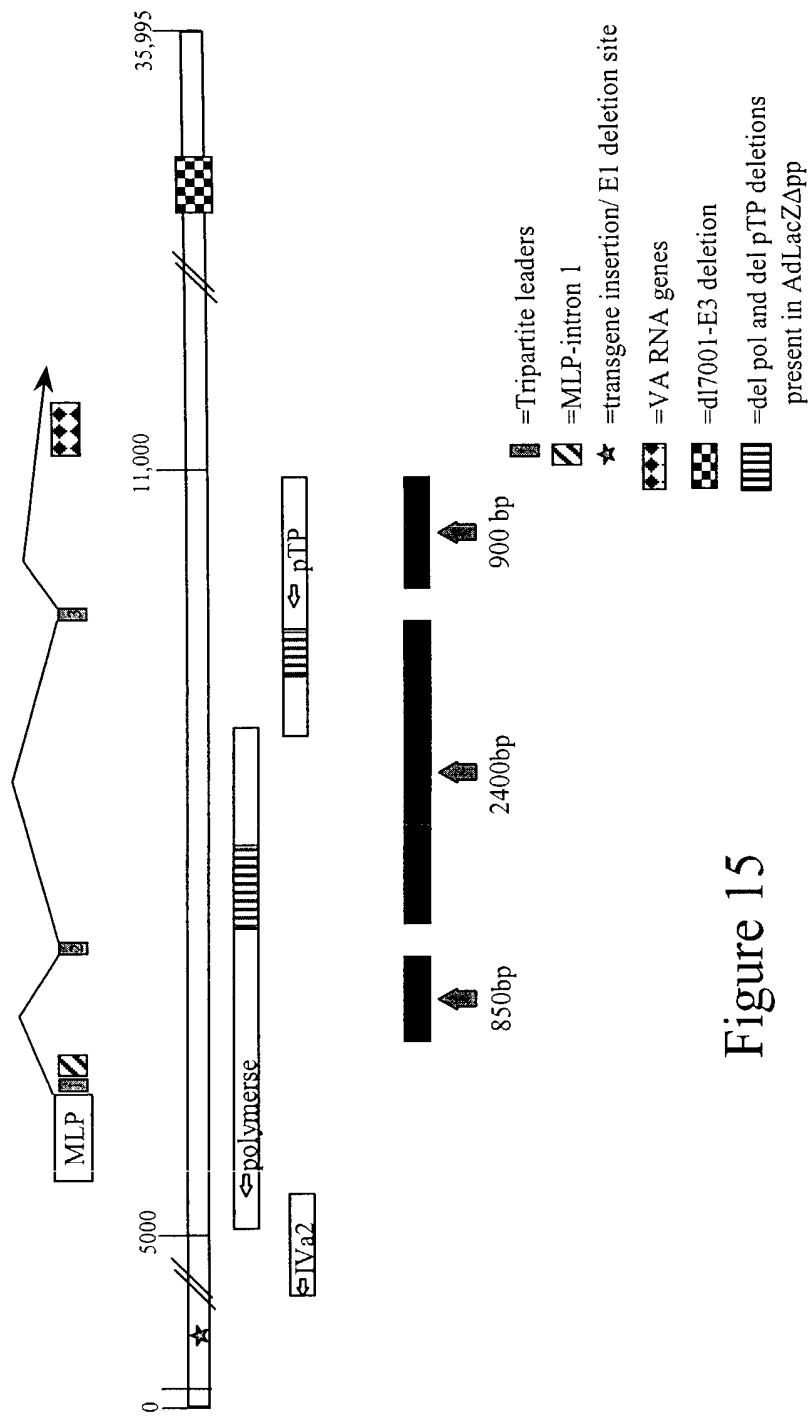- = dl7001-E3 deletion
- = del pol and del pTP deletions present in AdLacZΔpp
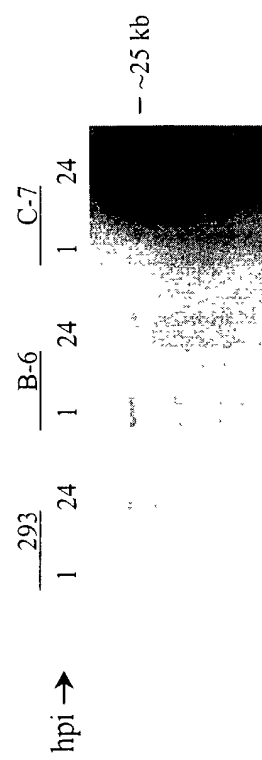
Figure 16
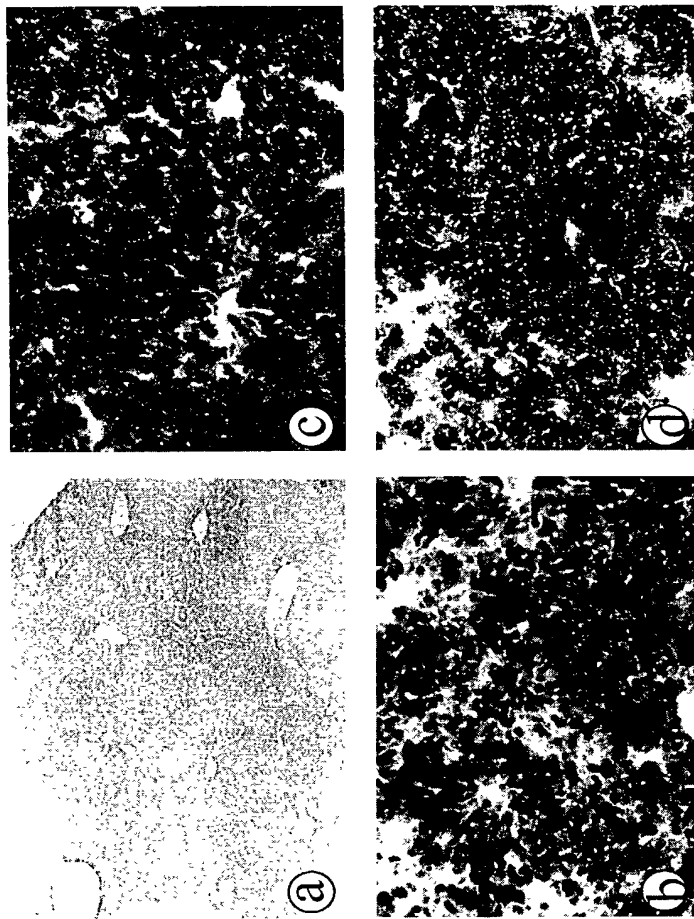
Figure 17

Figure 18

pAdAscL-ΔIVa2 shuttle plasmid(~14.2kb) Amp-Res

Linearization sites
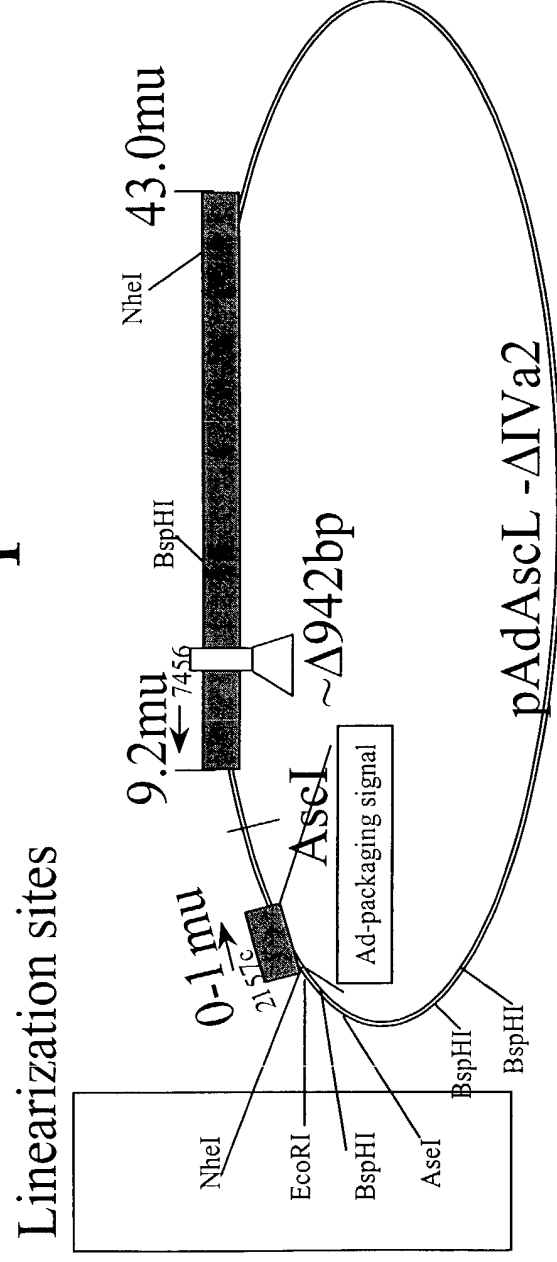
← primer site location of indicated primer

Figure 19

Figure 20

100K mRNA

1 2 3 4 5 6
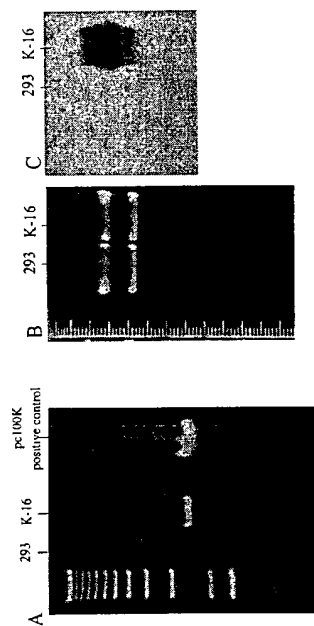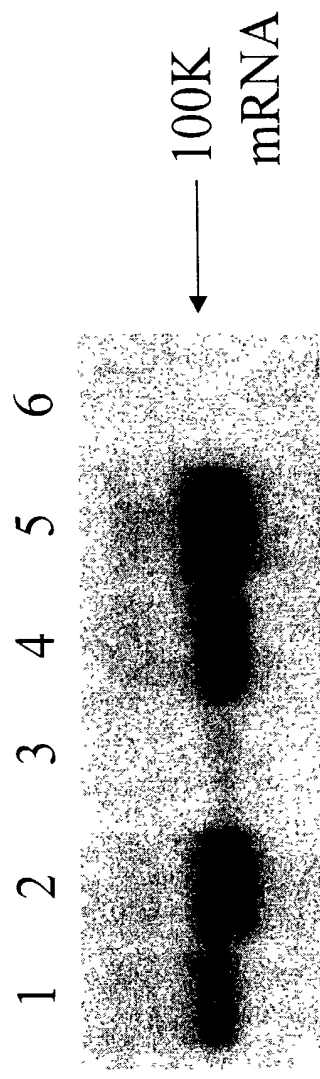
E1+pol+pTP+IVa2+100K= Lanes 1-4

E1+100K=Lane 5

E1=   Lane 6

Figure 21

Figure 22

FIG. 23

GAA activity
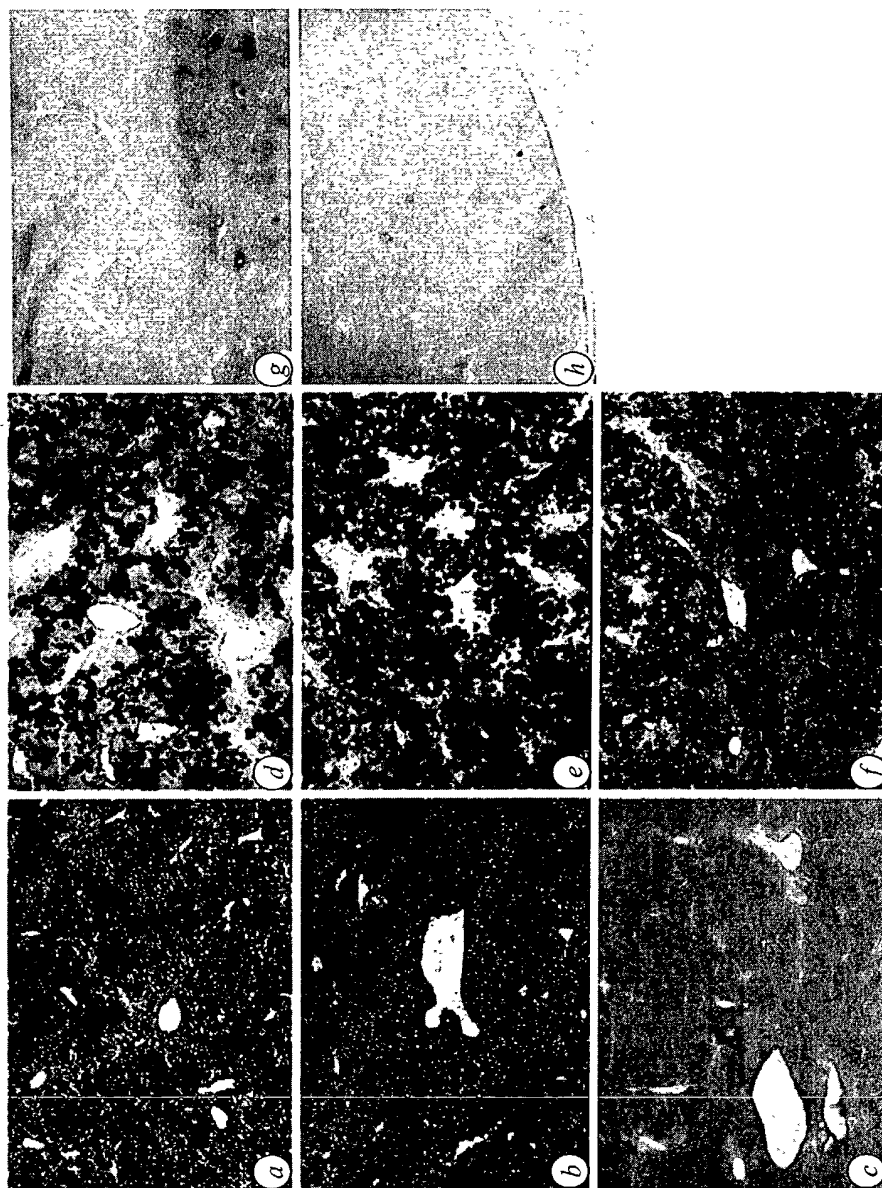nmol/hr/ml
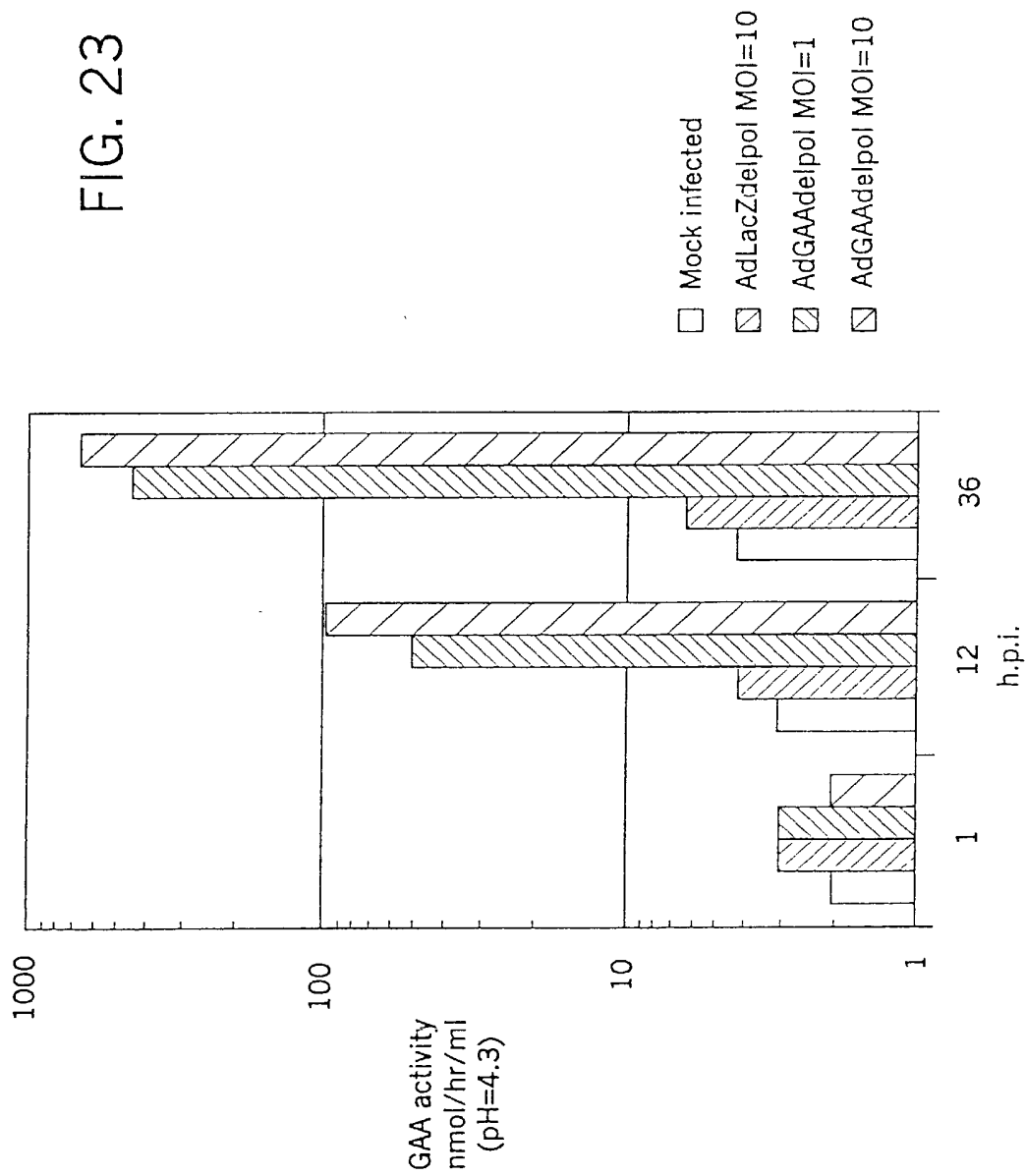(pH=4.3)

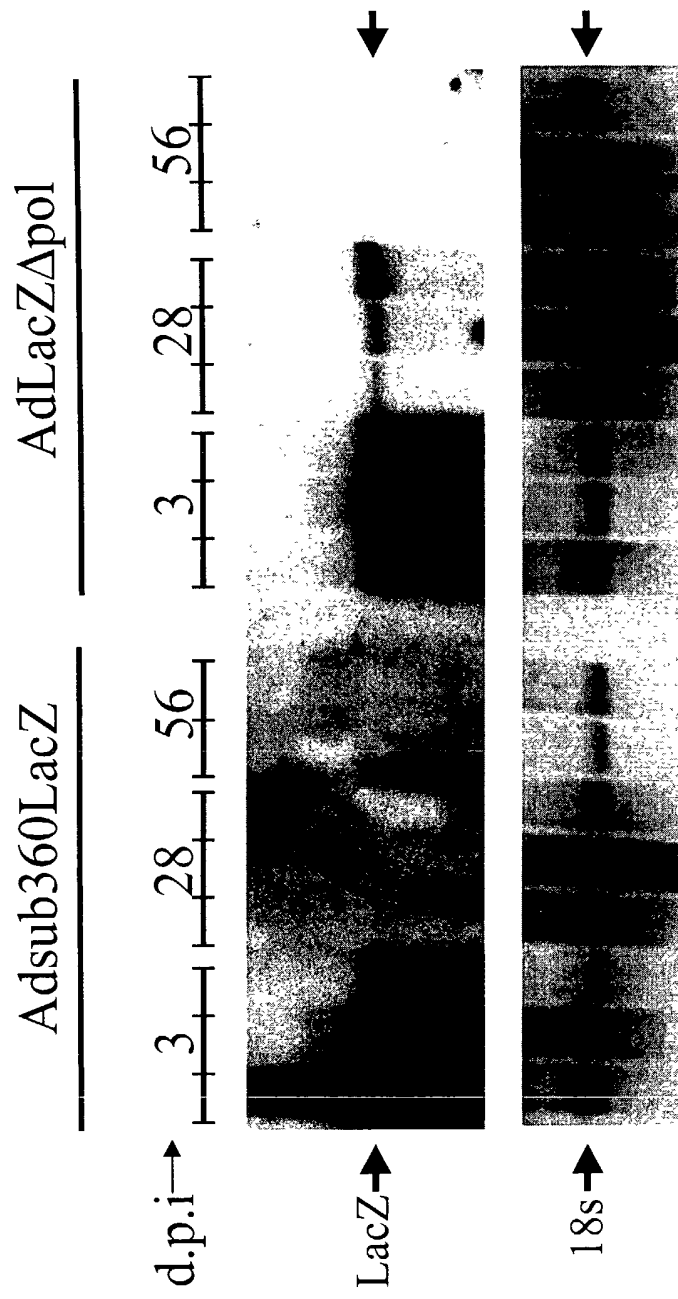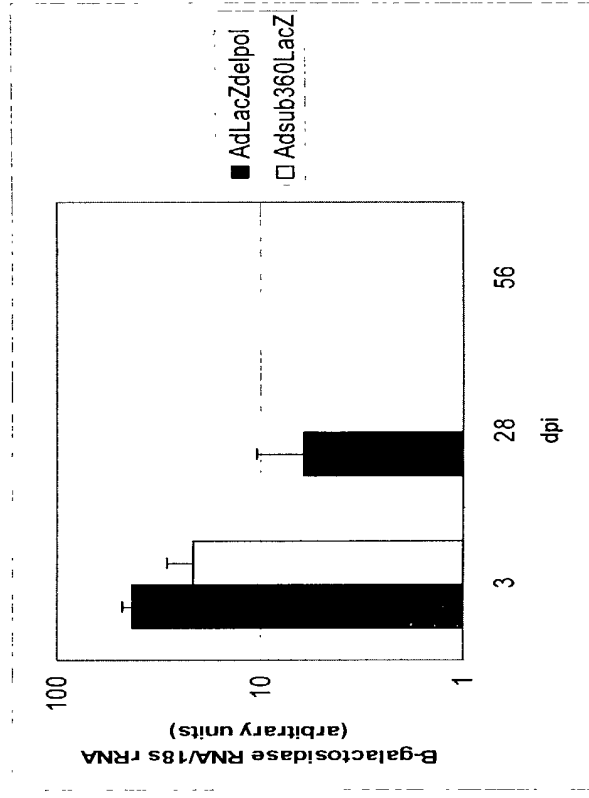□ Mock infected
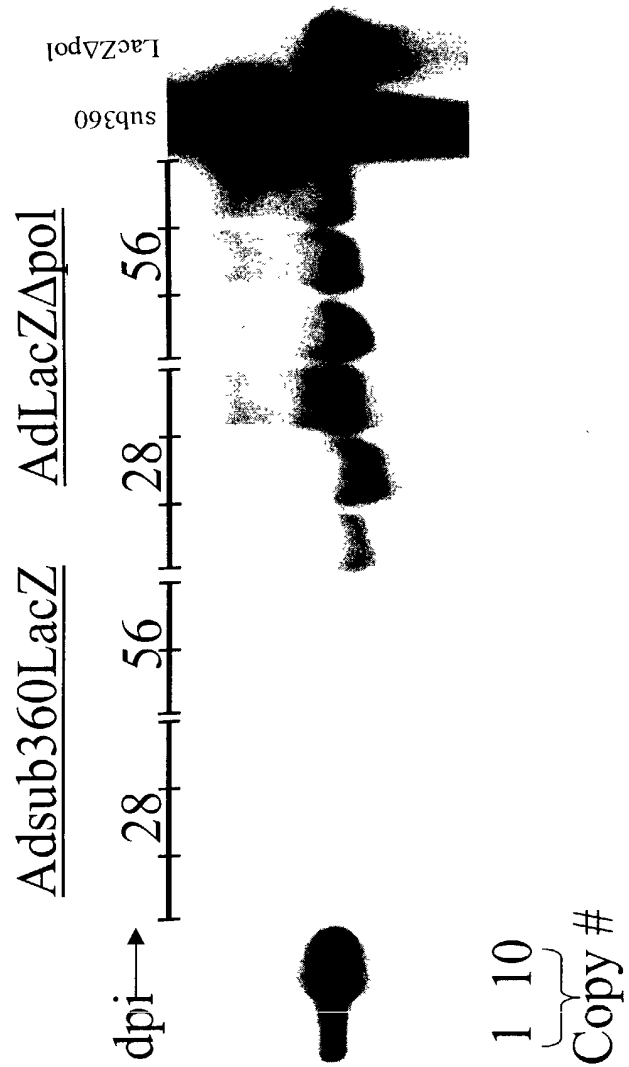▨ AdLacZdelpol MOI=10
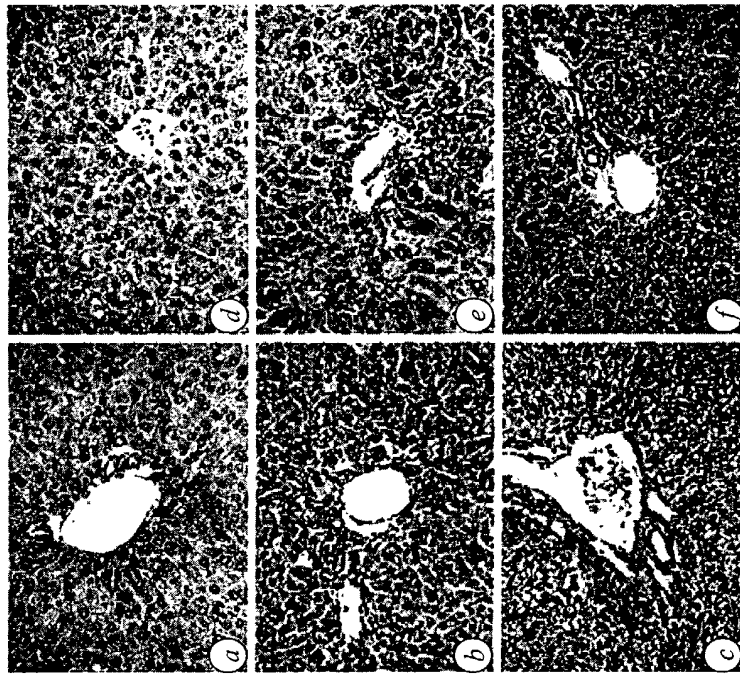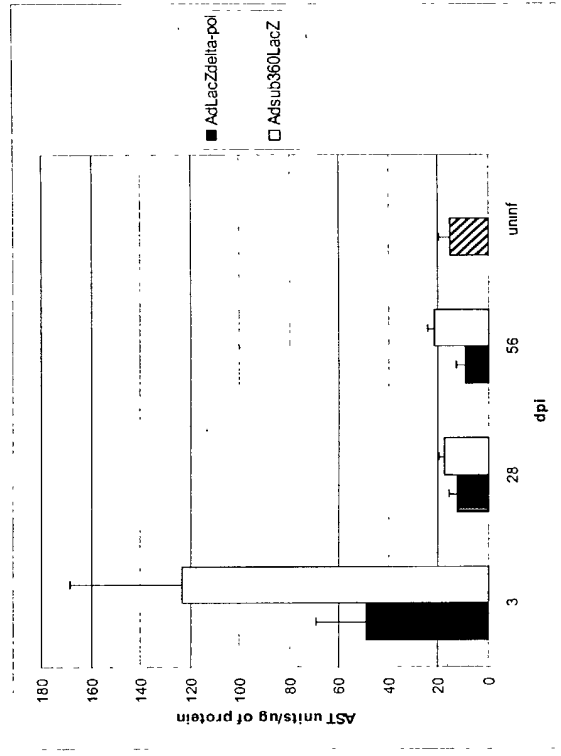▨ AdGAAdelpol MOI=1
▨ AdGAAdelpol MOI=10
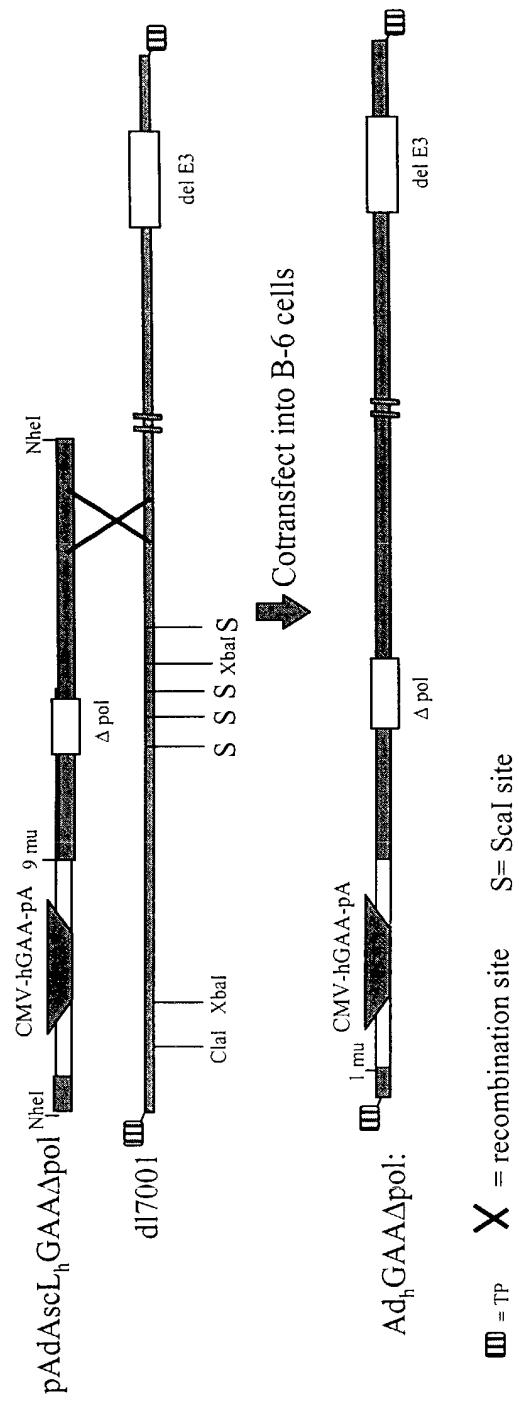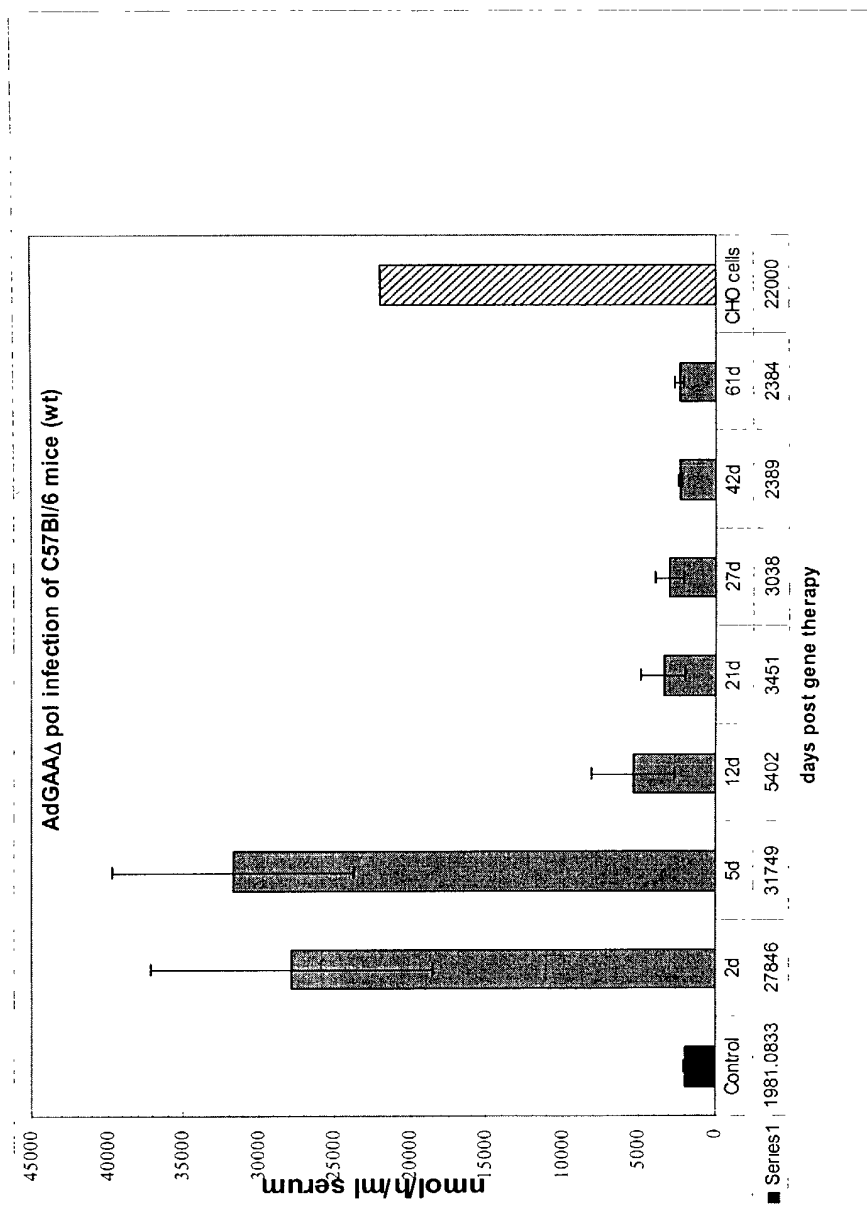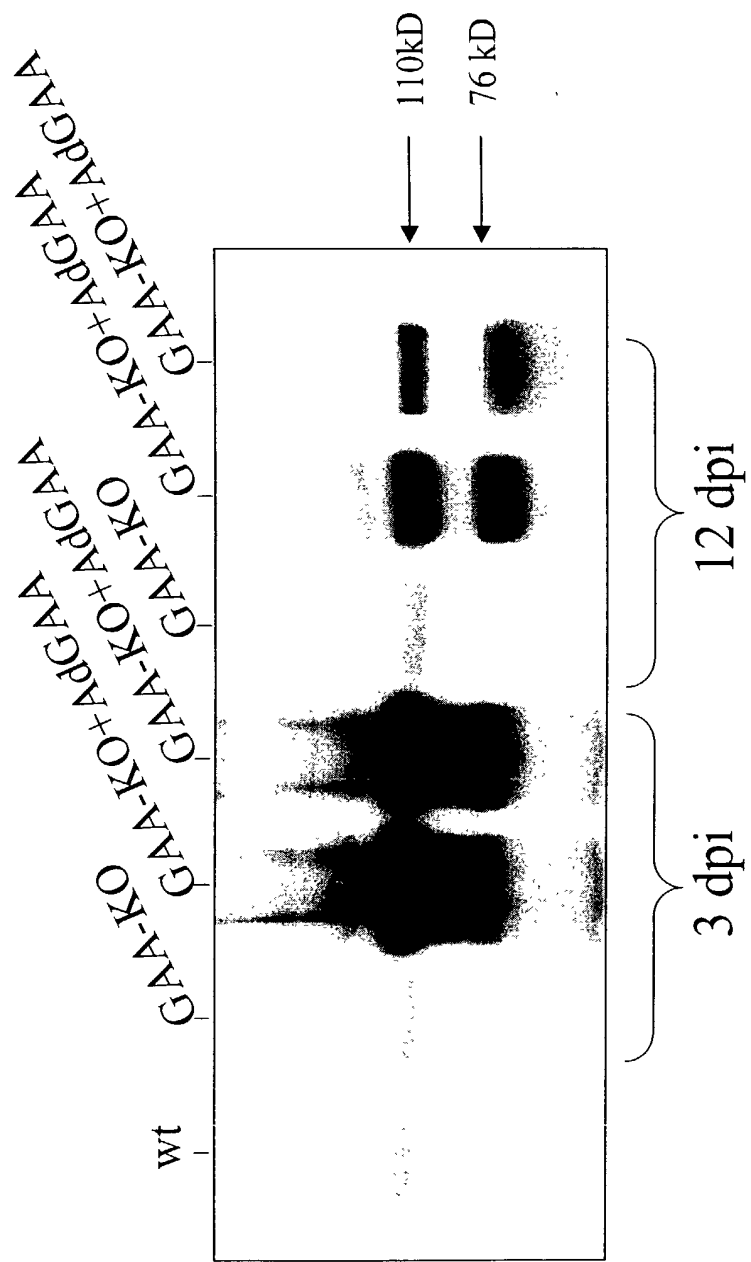
h.p.i.

Figure 24

Figure 25

Figure 26

Figure 27

Figure 28

Figure 29

Figure 31

Figure 32

Figure 33

Figure 34

Heart

AdhGAA-treated

Untreated

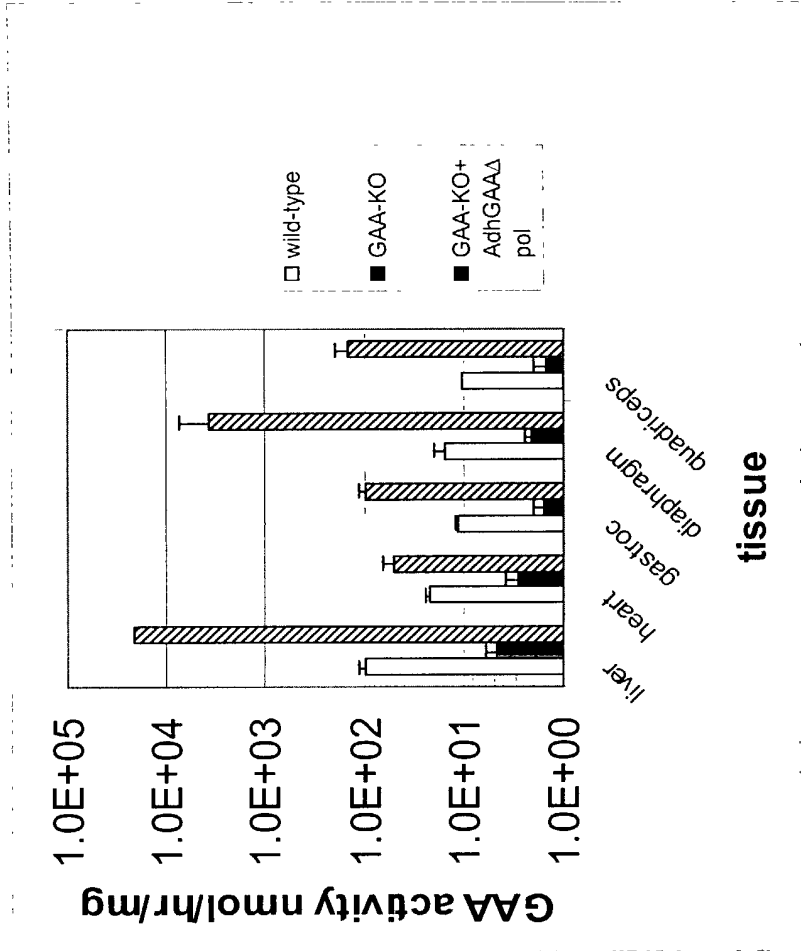Gastroc.

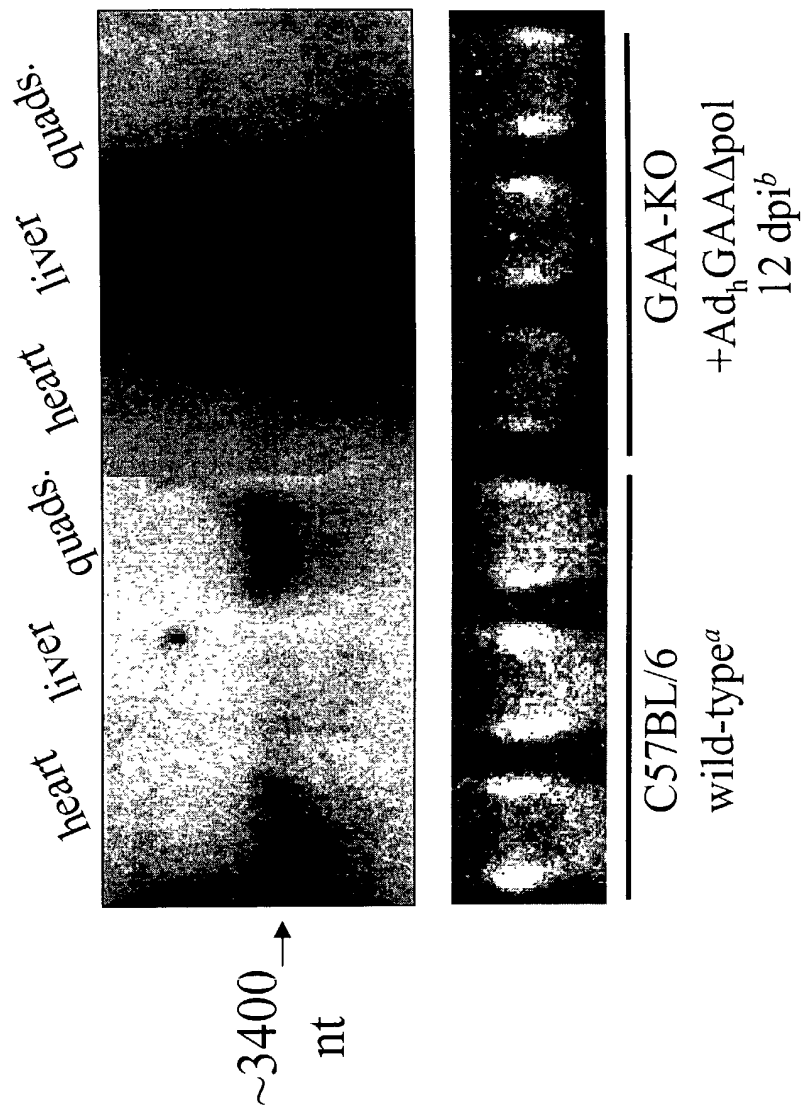Quads

Diaphragm

AdhGAA-4dpi

Untreated

Wild-type

AdhGAA-12dpi
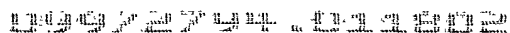
Figure 35

pAdAscL-ΔIVa2,Δpp-1.6kb:intermediate shuttle plasmid(~12.6kb) Amp-Res

Linearization sites
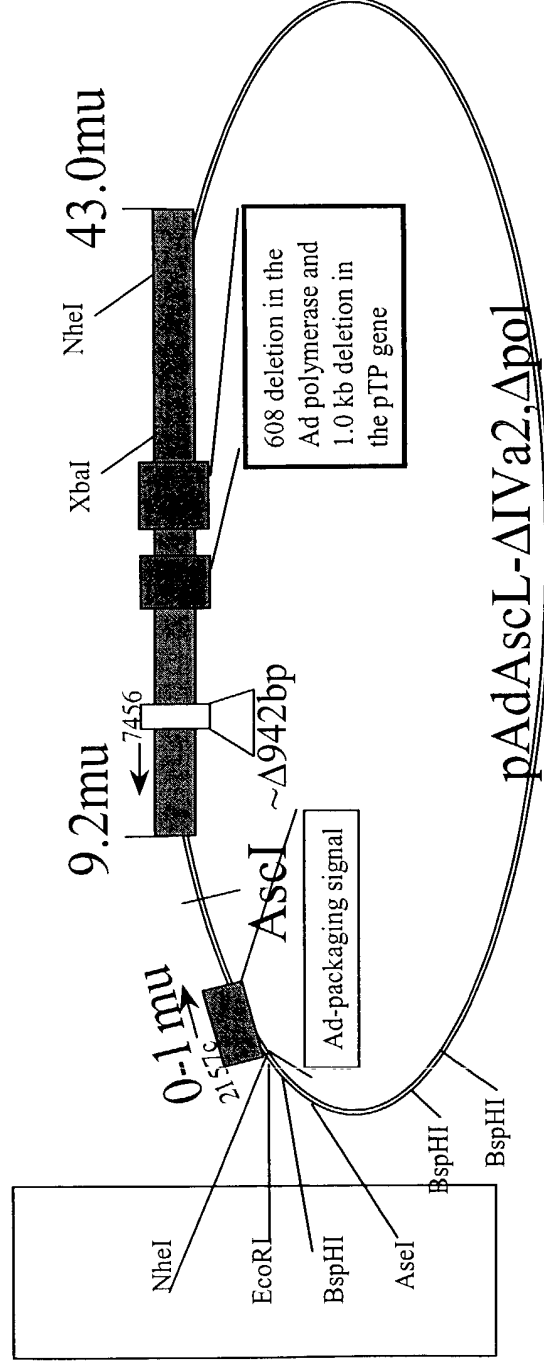
Figure 36

pAdAscL-ΔIVa2,Δpp-2.4kb shuttle plasmid(~11.8kb)

Amp-Res

Linearization sites

9.2mu

0-1 mu

21576

7456

43.0mu

NheI

XbaI

NheI

AscI

2.4 kb deletion in the Ad polymerase and pTP gene

~Δ942 bp deletion in IVa2+pol

Ad-packaging signal
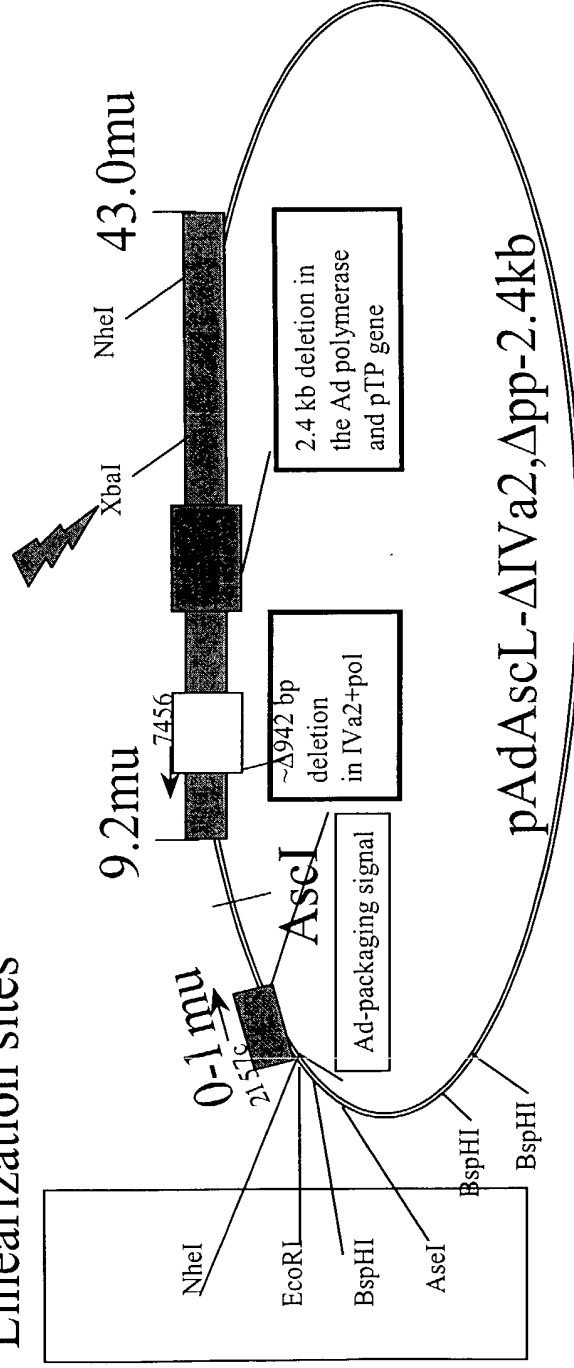
NheI

EcoRI

BspHI

AseI

BspHI

BspHI

pAdAscL-ΔIVa2,Δpp-2.4kb

← primer site location of indicated primer

Figure 37

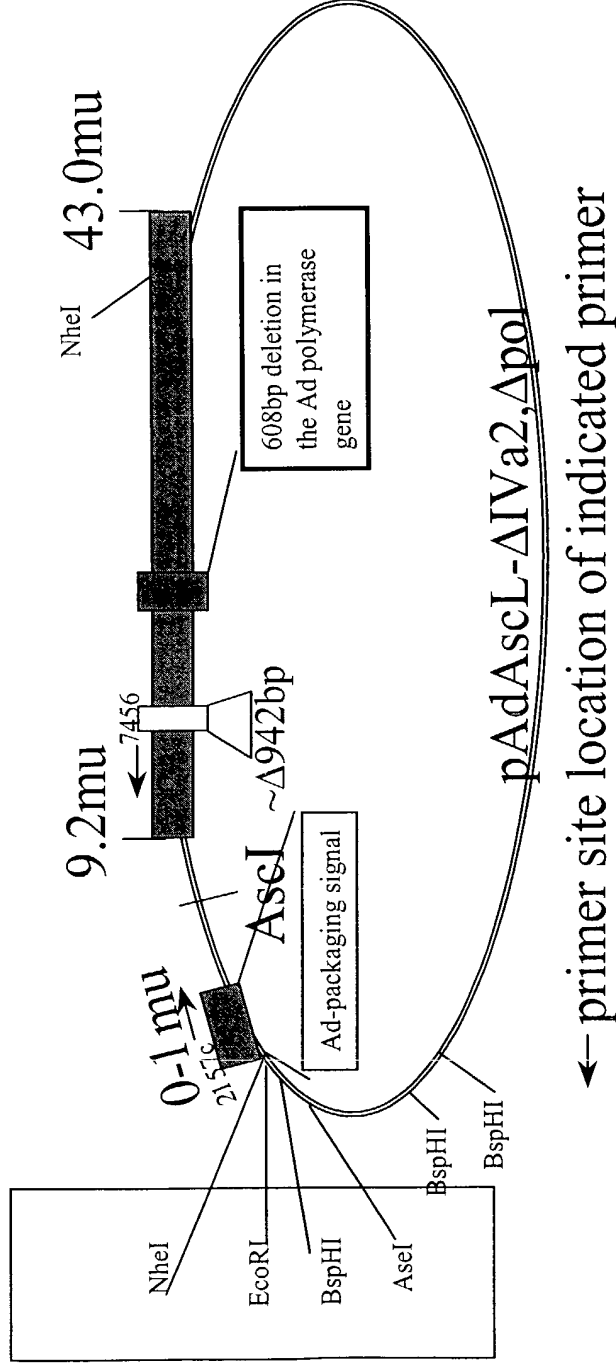# pAdAscL-ΔIVa2,Δpol shuttle plasmid(~13.6kb)
Amp-Res

Linearization sites

9.2mu

NheI   43.0mu

←7456

~Δ942bp

608bp deletion in the Ad polymerase gene

0-1 mu
21516

AseI

Ad-packaging signal

NheI

EcoRI

BspHI

AseI

BspHI

BspHI

pAdAscL-ΔIVa2,Δpol

← primer site location of indicated primer

Figure 38